

# RESTORE

A large-scale research initiative on  
ATMPs

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FONDAZIONE



# Presentation outline

1. What is a large scale research initiative
2. What is RESTORE (consortium and vision)
3. How the RESTORE strategic research agenda will be defined
4. What's next on RESTORE

# A long, participatory path towards the large-scale research initiatives 2030

Large scale research initiatives are 1-billion, 10-years investment by the European Commission and Member States focusing on specific scientific and technological challenges.

March 2019: we are here



1 Mar  
2019:

Start of the ATMP challenge - RESTORE preparatory action (12 months duration)

Spring  
2020:

Launch by the EC of two Large-scale Research initiatives 2021-2030

Feb-Apr  
2016:  
Public Consultation on potential challenges

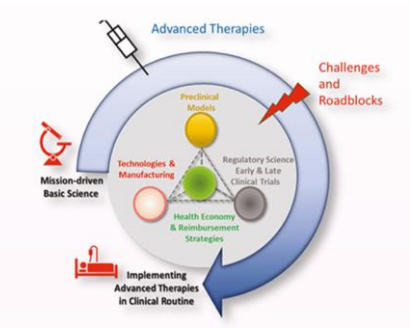
20 Feb  
2018:  
H2020 call for Preparatory Actions for challenge definitions – 1<sup>st</sup> stage

18 Sept  
2018:  
H2020 call for Preparatory Actions for challenge definitions – 2nd stage



# RESTORE

**RESTORE - Health by Advanced Therapies**  
(Advanced Therapy Medicinal Products and Biologised Medical Devices)



The long selection process:

- 30<sup>th</sup> of April 2016: **24 ideas** were received as a result of the public consultation
- 20<sup>th</sup> of February 2018: **32 proposals** were submitted at the 1<sup>st</sup> stage of the call H2020-FETFLAG-01-2018
- 31<sup>st</sup> of May 2018: **17 proposals** were admitted to the 2<sup>nd</sup> stage of the call H2020-FETFLAG-01-2018.
- 3<sup>rd</sup> of December 2018: **6 preparatory actions** were selected for funding, 2 per each of the 3 main areas: ICT and Connected Society; **Health and Life Sciences**; Energy, Environment and Climate change.
- 1<sup>st</sup> of March 2019: **the Restore preparatory action officially started!**

## Our Vision & Mission:

- The unifying goal of the RESTORE initiative is to make the transforming promise of **Advanced Therapies** (Cell & Gene therapies, Tissue-Engineering products, biologised Medical Devices) a reality through the development of new Advanced Therapies and their implementation in clinical practice to enhance the value-based outcome of patients.
- We are aiming at a future in 2030 and beyond where dozens of Advanced Therapy products “discovered & made in Europe” become world-market leaders, where Advanced Therapies are affordable and standard-of-care for many currently incurable diseases and finally, where every European citizen has access to Advanced Therapies that are required for his/her personalized treatment.
- Driving innovation, patient-centered clinical research, and adherence to high quality and ethical standards are central tasks of the RESTORE initiative.
- **RESTORE** health will become a game changer in health care within the next decade and will have a high impact on Europe's society and economy.

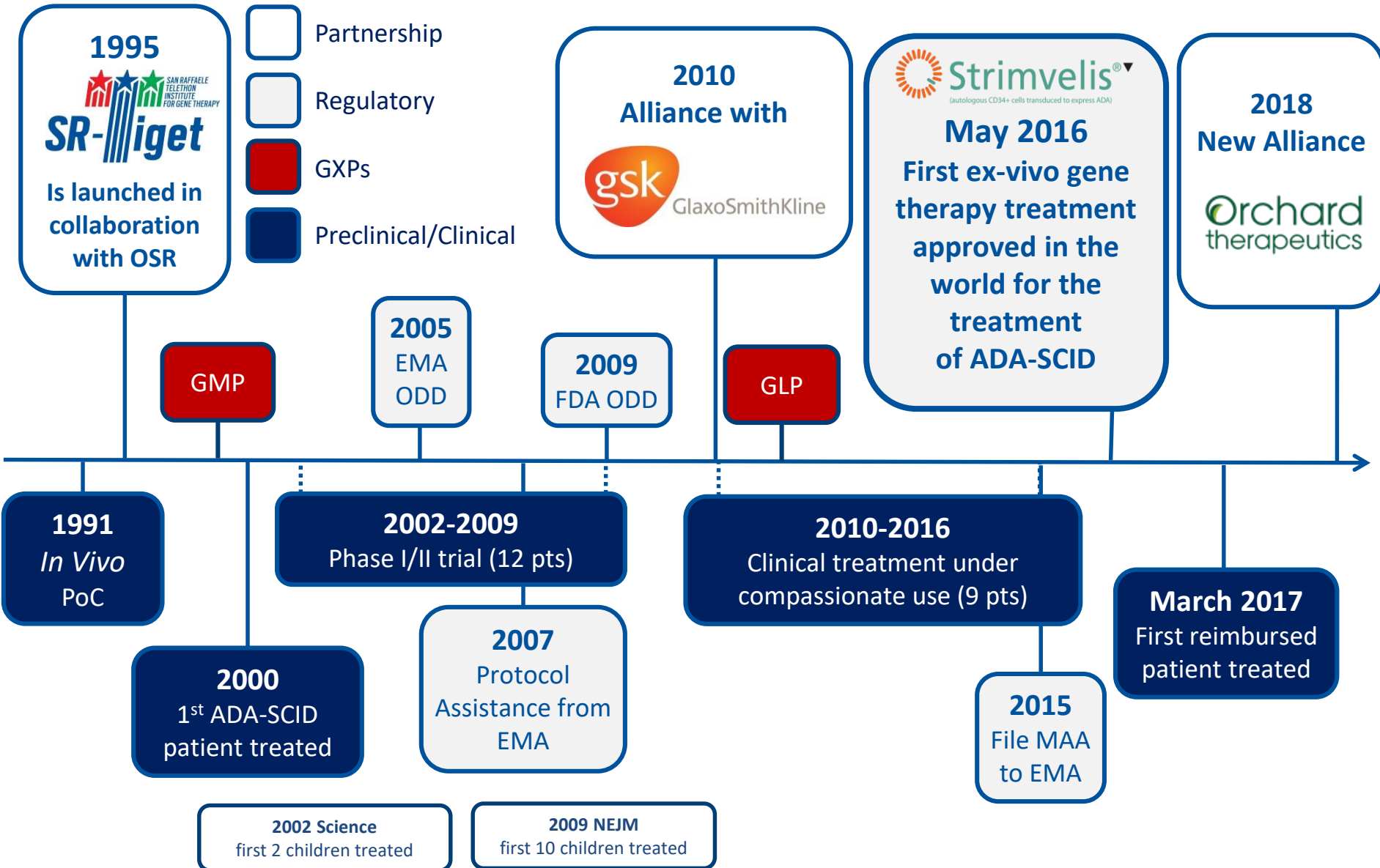
The issues and problems to be solved are:

- Overcoming the scattered research and development efforts across Europe
- Addressing the Science & Technology (S&T) challenges identified and tackling the major roadblocks that prevent implementation of ATMPs in clinical practice
- Supporting academic translational hubs to allow cost-effective de-risking and translation of product development for industry
- Establishing new educational paths dedicated to ATMPs

# The Restore consortium

1. **BCRT/Charité, Germany (Coordinator)** – *Hans-Dieter Volk, Petra Reinke, Mohamed Abou El-Enein*
2. University of Zurich, Switzerland – *Simon P. Hoerstrup, Maximilian Emmert*
3. Cell and Gene Therapy Catapult, United Kingdom – *Terri Gaskell, Michaela Sharpe*
4. TissUse GmbH, Germany – *Uwe Marx, Silke Hoffmann*
5. Pluristem LTD, Israel – *Racheli Ofir, Orit Hershkovitch*
6. Miltenyi Biotec GmbH, Germany – *Michael Apel, Mario Assenmacher*
7. INSERM – Institut National de la Santé et de la Recherche, France – *Annelise Bennaceur-Griscelli, Ali Turhan*
8. Innovation Acta S.r.l., Italy – *Gabriela Dessole, Natascia Cannella*
9. Fondazione Telethon, Italy – *Stefano Benvenuti, Michela Gabaldo*
10. University of Minho, Portugal – *Rui Reis, Nuno Neves*

# Strimvelis<sup>®</sup>: an example of Telethon's impact in the ATMPs field



# Key topics – basic science and technology development

WG	Research Platform	Topic	Lead	Co-lead
1	Manufacturing	(modified) cells	Pluristem	Miltenyi, INSERM
2	Manufacturing	Tissue Engineering & Composite Products	Zurich	Minho
3	<b>Manufacturing</b>	<b><i>In vivo</i> gene therapy</b>	<b>Telethon</b>	
4	Manufacturing	Pluripotent stem cells	Inserm	
5	Manufacturing	Ex vivo gene delivery/editing	Catapult	Miltenyi
6	Pre-clinical model systems	<i>In vitro</i> and <i>in vivo</i>	TissUse	Inserm
7	New Clinical Applications	Endogenous regeneration	Charité	Pluristem
8	<b>New Clinical Applications</b>	<b>Replacement – gene replacement, silencing, editing</b>	<b>Telethon</b>	<b>Zurich</b>
9	New Clinical Applications	Cancer	Miltenyi	
10	Clinical Research	Regulatory science, refined translation, early HTA & early clinical trials	Charité (M.AE-E)	Catapult
11	<b>Clinical Research</b>	<b>Pivotal Clinical Trials and Marketing Authorisation</b>	<b>Telethon</b>	<b>Pluristem</b>
12	<b>Implementation</b>	<b>Post-Trial Follow-up and Data Warehouse &amp; Long-term follow-up</b>	<b>Miltenyi</b>	<b>Telethon</b>
13	Implementation	Implementation of new Advanced Therapies into clinical routine	Charité (M.AE-E)	Catapult
14	<b>Implementation</b>	<b>Valuation and innovative reimbursement models for new Advanced Therapies</b>	<b>Telethon</b>	<b>Pluristem</b>

For each topic a Scientific Working Group will define the main challenges and the research strategy for the next years.

The WGs will meet for the first time in Berlin on the 6<sup>th</sup> and 7<sup>th</sup> of May 2019.



# RESTORE's expected impact

## Main Target:

a) European and national life-science ecosystem

b) Citizens

c) Industry (in particular Pharma and Biotech)

d) Scientific community

e) Healthcare providers and payers

## Key Impact

Europe will remain a relevant player in the field and not just a payer of treatments developed in the USA and Asia

Better treatments for patients with highly unmet medical need

New business opportunities to keep up with the global trend

Innovation in cutting edge fields (eg. gene editing)

Better treatments, paradigm shift from chronic treatment to cure

# First list of touchpoints

## March

- TEDDY Conference in Madrid
- Lombardy LS cluster

## April

- ESGCT Spring School 2019
- ASGCT Congress 2019

## May

- World Advanced Therapies & Regenerative Medicine Congress 2019
- IRDiRC Consortium Assembly (?)

## September

- EJP general assembly (?)

## October

- ESCGT congress 2019

## November

- ICPeMed conference in Madrid
- Telethon scientific convention
- Nature Conferences - Advanced Cell Therapies and Tissue Engineering

## TBD

- Workshop in Brussel focusing on Regions (with Toscana Life Science)
- Workshop in Brussel focusing on companies (with Assolombarda)
- Workshop in Brussel focusing on patients (with EURORDIS)

Thanks

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